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TKT SCIENTISTS DELIVER ERYTHROPOIETIN (EPO) BY GENE THERAPY; NOVEL GENE ACTIVATION TECHNOLOGY ELIMINATES NEED FOR EPO LICENSE

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SAN FRANCISCO, Jan. 12 /PRNewswire/ -- Transkaryotic Therapies Inc. (TKT), a Cambridge, Mass.-based company specializing in non-viral gene therapy products, today reported at Hambrecht & Quist's 12th Annual Life Sciences Conference that they have succeeded in obtaining long-term expression of erythropoietin (EPO) in animal models. EPO is an expensive protein used primarily for the treatment of chronic anemia caused by kidney disease. Patients undergoing dialysis currently receive weekly injections of genetically engineered EPO at a cost of approximately $6,000 per year, and once started, EPO treatment often lasts for the rest of the patient's life.

"TKT's gene therapy product is based on treating these patients with a single administration of cells which have been genetically engineered to produce EPO, an approach that could represent a superior therapy at significantly lower costs," said Richard F. Selden, M.D., Ph.D., TKT's founder and chief scientific officer, at the H&Q conference. In experiments on laboratory animals, Selden reported that TKT's scientists have demonstrated that the implantation of normal skin cells modified by the insertion of the EPO gene results in the long-term production of therapeutic levels of EPO for a period of one year. The experiments are continuing to determine whether the procedure will last for the entire lifetime of the animals.

"This approach represents a major potential advantage over existing therapy, yet we would be unable to commercialize it in the U.S., unless we were to obtain a license to the EPO gene patent from Amgen or Johnson & Johnson," explained K. Michael Forrest, TKT's president and chief executive officer. "Fortunately, our research scientists have come up with a novel way of activating the existing EPO gene in normal cells in a fashion which absolutely eliminates the need to get a license on the gene patent," he said.

TKT's proprietary gene activation technology involves the surgically precise modification of a patient's cells. A piece of DNA is inserted adjacent to the natural EPO gene in normal cells -- this piece of DNA represents a genetic "switch" that turns on EPO production in the cells. The technology is an extension of the work on the genetic modification of normal human cells developed at TKT over the past five years. The technology can be applied to the activation of essentially any human gene, the company noted.

TKT is currently engaged in pre-clinical studies of the company's EPO gene activation product for the treatment of severe anemia and is discussing additional gene activation targets with corporate partners.

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